

Engineered iPSC for therapy of limb girdle muscular dystrophy type 2B

Grant Award Details

Engineered iPSC for therapy of limb girdle muscular dystrophy type 2B

Grant Type: Early Translational IV

Grant Number: TR4-06711

Project Objective: Test feasibility of autologous corrected iPSC derived satellite cells to improve disease severity in a model of limb girdle muscular dystrophy 2b, LMGDM2B.

Investigator:

Name:	Michele Calos
Institution:	Stanford University
Type:	PI

Disease Focus: Muscular Dystrophy, Pediatrics, Skeletal/Smooth Muscle disorders

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: \$1,876,253

Status: Closed

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 3

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Grant Application Details

Application Title: Engineered iPSC for therapy of limb girdle muscular dystrophy type 2B

Public Abstract: Limb girdle muscular dystrophy type 2B (LGMD 2B) is a form of muscular dystrophy that leads to muscle degeneration and disability. In LGMD 2B, a vital muscle protein is mutated, and its absence leads to progressive degeneration of muscles in the body that are needed for mobility. To create a therapy, we will provide a new supply of stem cells that carry the missing protein that is lacking. These cells will be delivered to the body in such a way that they will engraft into the muscles and produce new, healthy muscle tissue on an ongoing basis.

We now possess methods to create stem cells that can become muscle cells out of adult skin cells by a process known as "reprogramming". By reprogramming adult cells, together with addition to them of a correct copy of the gene that is mutated in LGMD 2B, we will create stem cells that have the ability to create new, healthy muscle cells in the body of a patient. This is the type of strategy that we are developing in this proposal. The corrected muscle stem cells will be transplanted into mice with LGMD 2B, and the ability of the cells to generate healthy new muscle tissue and increased muscle strength will be evaluated.

This project could lead to a new stem cell therapy that could improve the clinical condition of LGMD 2B patients. If we are successful with this disease, similar methods could be used to treat other degenerative disorders, and perhaps even some of the degeneration that occurs during muscle injury and normal aging.

Statement of Benefit to California: The proposed research could lead to a stem cell therapy for limb girdle muscular dystrophy type 2B (LGMD 2B). This outcome would deliver a variety of benefits to the state of California. There would be a profound personal benefit to the Californians affected directly or indirectly by LGMD 2B.

Progress toward a cure for LGMD 2B is also likely to accelerate the development of treatments for other degenerative disorders. The most obvious targets would be other forms of muscular dystrophy and neuromuscular disorders. Muscle injury, and even some of the normal processes of muscle aging, may be treatable by a similar strategy.

An effective stem cell therapy for LGMD 2B would also bring economic benefits to the state by reducing the huge burden of costs associated with the care of patients with long-term degenerative disorders. Many of these patients would be more able to contribute to the workforce and pay taxes.

Another benefit is the effect of novel, cutting-edge technologies developed in California on the business economy of the state. Such technologies can have a profound effect on the competitiveness of California through the formation of new manufacturing and health care delivery facilities that would employ California citizens and bring new sources of revenue to the state.

Therefore, this project has the potential to bring health and economic benefits to California that are highly desirable for the state.

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